***VIA EMAIL AND POSTED PUBLICLY***

April 8, 2019

The Right Honourable Justin Trudeau, PC, MP
Prime Minister of Canada
80 Wellington St.
Ottawa, Ontario K1A 0A2

**Subject:** **Patients say "NO" to unjustifiable new regulations that will obstruct the entry of new medicines to Canada**

Dear Prime Minister Trudeau,

We apologize in advance if the tone of this letter conveys less than the utmost respect. What you are actually sensing is our extreme frustration and indeed fear for Canadians who rely on access to patented medicines.

Specifically, we are concerned about your government's proposed changes to the Patented Medicines Regulations, which would overhaul how the Patented Medicine Prices Review Board (PMPRB) sets maximum (non-excessive) prices for patented drugs in Canada.

As two of the three patient members appointed to the PMPRB Steering Committee on Guidelines Modernization (SC), we feel strongly that our responsibility is to ensure the needs of the millions of Canadian patients are adequately reflected in the proposed regulatory changes. Outside of the Steering Committee process, our organizations have also consulted with patients and the public to develop extensive and detailed feedback over the past two years. It is not an overstatement to say that *none* of our input has been addressed or resulted in any substantive changes in the Health Canada and PMPRB proposals.

The Steering Committee process has been even more frustrating and ultimately infuriating. As patient members, we have had to work diligently to understand the potential impact of proposed changes, to actively participate in the Steering Committee discussions, and to provide oral and written input.

It was clear from the start that none of the SC members had any real role in steering guidelines development. Instead, we were limited to responding to policy directions already decided by PMPRB staff. They provided limited information (data and analyses) to validate the policies and guidelines that were presented and they rejected the opportunity to propose any alternatives or options for achieving the ultimate goal (that is, ensuring non-excessive and internationally compatible prices). One can see clearly in each set of meeting minutes and subsequent documents the limited scope of discussions and minimal impact on the original policies and guidelines.

The experience with the Technical Working Group (TWG), set up to provide scientific and economic rationale for the proposed regulations and criteria for implementation has been equally if not more maddening. At the conclusion of their five-to-six-month work, they produced a report that basically said they were unable to substantiate the premises for the PMPRB guidelines. They were also unable to make any recommendations for changes, primarily because they did not have access to the necessary information, or they were limited by their terms of reference.

Steering Committee members were presented with these "non-recommendations" by the Chair of the TWG in a one-hour teleconference and then subsequently asked to provide opinions on a series of questions that are essentially unanswerable because we do not have the data or analyses on which to make any informed judgments. We choose not to provide arbitrary answers that will perpetuate the myth that there was ever a Steering Committee. We have been no more than passengers in a vehicle on a pre-set course that is now going "over the cliff" (think: crash test dummies).

In summary, based on what we do know from the case studies prepared by the PMPRB staff, the impact of the proposed regulations will be to drive initial list prices to levels so low (about 30% to 90% below most international list prices) that no company will bring new drugs to Canada. This is not an idle threat; it is just the experience of countries (mostly low- and middle-income ones) that have extremely depressed prices.

We all want to have access to medicines at affordable prices. However, these proposed changes by the PMPRB will mean that many new therapies will not be available in Canada.

**It will be unethical, unjustifiable, and unfair but nevertheless inevitable that Canadian patients will be denied breakthrough, lifesaving, and even incrementally better medicines.**

Instead of responding to the questions, given the lack of real consultations by both Health Canada and the PMPRB staff, we feel compelled to take this opportunity to share our questions and our responses that will explain why the PMPRB reform needs to be fundamentally reconsidered.

***Question 1: What is the impact on patient access to new medicines?***

The proposed new federal regulations would mandate drug prices so low that we are concerned that companies will not launch new drugs in Canada, or they will wait until after they have been available for years, everywhere else.[1]

[1] Steering Committee on Modernization of Price Review Process Guidelines, specifically, the Guideline Modernization: Case Studies and Proposed Application of PE and Market Size Factors to Category 1 Drugs (http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1378&lang=en).

***Question 2: Who is at risk?***

Patients with terminal cancer who have failed all previous therapies; patients with rare conditions who have no previous therapies; patients for whom cell and gene therapy may offer a rescue; and patients with chronic conditions whose current therapies are losing their effectiveness.

***Question 3: Is this unethical?***

The proposed regulations instruct the PMPRB to target those therapies with the greatest unmet need and those that offer potentially the biggest improvement over existing therapies. Moreover, the PMPRB states, "…that its mandate is to protect consumers from excessive pricing, and not to ensure that products are launched into the market."[2]

***Question 4: What is unjustifiable about the proposed reforms?***

The proposed regulations will define how new ceiling prices will be calculated based on a number of factors, mostly economic, but not determined or supported by science, economic analysis, modeling, best practices, or even the experience of other countries. We are not aware of any other country in the world that proposes to regulate both the public list price and confidential price for every transaction in an entire jurisdiction, and outside of a reimbursement or pharmacare program.

***Question 5: Are the "cost-effectiveness" thresholds valid?***

In July 2018, the PMPRB Technical Working Group was set up to validate or recommend changes to the criteria, metrics, and criterion thresholds, along with other cost-effectiveness considerations. The TWG presented its report in March 2019 to the PMPRB and the Steering Committee, and concluded it was mostly unable to validate the PMPRB's proposed criteria and also unable to provide solid recommendations. The TWG cited the lack of "necessary data" to even model how many drugs would be classified for increased scrutiny.[3] The TWG was "unanimous in considering the empirical evidence with respect to Canadian estimates of supply-side thresholds to be uncertain."[4] This uncertainty is compounded by the fact that the only study cited to estimate this threshold was not peer reviewed, the research was not primarily based on Canadian data and some of the variables employed were not relevant to Canada.[5]

In fact, the TWG found that they could not definitively address most of the recommendations posed in their Terms of Reference, citing "limitations in the empirical and theoretical literature."[6]

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[2] Technical Working Group report p. 53.
[3] TWG report p. 18.
[4] TWG report p. 23.
[5] Assessing health opportunity costs for the Canadian health care systems (Report by the University of York) (http://www.pmprb-cepmb.gc.ca/CMFiles/Consultations/new_guidelines/Canada_report_2018-03-14_Final.pdf).
[6] TWG report p. 28.

In the 52-page TWG Final Report, the words "uncertain" or "uncertainty" are used in reference to the evidence, analyses, findings, and/or recommendations exactly 100 times.

***Question 6: Why are the proposed regulations unfair?***

The predominantly economic criteria, however inexact, will be applied equally across all therapies without regard for disease severity, rarity, treatment options, or other factors.

Indeed, the TWG recognized that there should be equity weights applied but did not have the information (knowledge base) to do so. "Characteristics that are often found to be important in empirical studies include severity of illness (particularly the presence or otherwise of life threatening or progressively chronically debilitating illness), the availability of active treatment alternatives, the prevalence of disease, the type of health gain (such as a reduction in pain), and the magnitude of health gain. These factors are often found to interact with one another, and so should not be considered independently. In the opinion of this member, greater empirical work is needed to fully understand these interactions and the 'weights' that would be put on each characteristic." [7] However, the TWG could not make a recommendation as to how equity weights could be implemented due to "limitations in the existing theoretical and empirical evidence base."[8]

That means, all other things being equal, the "maximum allowed cost" to provide "one additional year of life" for (1) a six-year-old with progressive neuromuscular disease, (2) a 42-year-old with metastatic breast cancer, and (3) an 81-year-old with well-controlled Type 2 diabetes is exactly the same. You can guess which medicines will meet the Canadian price threshold, and which will not.

***Question 7: Will the proposed regulations and the subsequent "ceiling price setting" result in lower Canadian prices for new medicines?***

The proposed switch in the basket of reference countries, including the dropping of the highest public price comparators (USA and Switzerland) and inclusion of lower-price countries could, in fact, result in lower public list prices. That means any payer or consumer purchasing at list price may get a lower transparent price.

However, we have no good analyses or data that can predict the potential impact of these changes on net prices overall. It may make no difference to all other payers who currently negotiate starting from a list price based on a cost-effectiveness assessment.

Even if the starting price were lower, there are no models to suggest that the final negotiated price would be different, especially since the public funders are negotiating collectively. The negotiated prices are confidential and may include risk-sharing agreements, rebates, and patient

[7] TWG report p. 28.
[8] TWG report p. 29.

support programs. Private payers and their plan sponsors who don't negotiate prices may benefit with a lower list price.

What will be different with this proposal is to use the net confidential prices to determine maximum prices for subsequent competitors and to apply that information to re-benching of therapeutic classes. Because every other country follows the current practice of holding negotiated prices confidential, Canada would show a lower list price, and this will inevitably deter companies from coming to Canada until they have negotiated their drug prices elsewhere. So, Canada may not, in the end, pay less than other counties; they will only get their drugs years later than other countries.

***Question 8: Will the proposed regulations result in lower global prices for new medicines?***

There will probably be no impact on global prices unless the rest of the developed world decides to publish their negotiated prices. Transparent pricing, in the long run, is probably a better approach, but Canada cannot go it alone. If we really want changes, we have to work collectively, especially with other OECD countries.

**Question 9: Why are we reforming the PMPRB *and* creating the Canadian Drug Agency**

Finally, your recent Budget announced the creation of a transition office for a new Canadian Drug Agency, which has as one of its objectives, saving Canadians $3 billion annually. The changes to the PMPRB and the Patented Medicines Regulations should be considered in this context. The proposed unfeasible, unfair and unjustified regulations would simply become a barrier to new medicines that the Canadian Drug Agency will rely on to complete the proposed comprehensive formulary.

In summary, we are asking you and your government to reconsider the changes to the Patented Medicines Regulations. Once the regulations are finalized, we are particularly concerned about how the PMPRB – an arm's length agency – will implement the changes. The PMPRB has demonstrated that it intends to implement them in a way that will obstruct and slow patient access to needed medicines.

Given the lack of any real consultations on the PMPRB Guidelines and the Patented Medicines Regulations, we are counting on your leadership to direct your officials to undertake meaningful consultations with patients in the context of your national pharmacare proposal. Done right, consultations on a new national pharmacare program could lead to affordable and appropriate access to medicines that patients need to be well and, in many cases, survive.

Please do not hesitate to reach out to either of us if you require further information regarding our position on this matter.

Sincerely,

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